

# EXHIBIT 3

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**FDA Grants Orphan Drug Designation to NS-065/NCNP-01 for the Treatment of Duchenne Muscular Dystrophy**

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January 16, 2017

R & D

### FDA Grants Orphan Drug Designation to NS-065/NCNP-01 for the Treatment of Duchenne Muscular Dystrophy

Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto City; President, Shigenobu Maekawa) announced today that the Food and Drug Administration (FDA) has granted Orphan Drug Designation to NS-065/ NCNP -01※1 which is being developed for the treatment of Duchenne Muscular Dystrophy (DMD) in patients who are amenable to exon 53 skipping in the United States (US).

The Orphan Drug Designation is issued to drugs which are intended for rare diseases that affect fewer than 200,000 people in the US or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment. Orphan drug designation provides for a seven-year marketing exclusivity period as well as certain incentives including tax breaks.

DMD is an inherited muscle disorder with the highest incidence that is limited to male children. It causes a severe loss of muscle power due to a deficiency of normal dystrophin, a protein involved in constructing the framework of muscle cells. This deficiency is caused by a mutation of the dystrophin gene. Because there is no effective treatment for DMD other than steroids, the development of an effective new treatment is desired.

NS-065/NCNP-01 is a morpholino antisense oligonucleotide, which is expected to improve muscle function by skipping a part of the genetic information of the dystrophin gene. A Phase 2 clinical study started in March 2016 in the US, and Fast Track Designation was granted by the FDA in October 2016. "SAKIGAKE designation"※2 of the Ministry of Health, Labor and Welfare in Japan was granted in October 2015.

The clinical study in the US is being conducted by NS Pharma, Inc. (Headquarters, New Jersey, US; President, Masato Matsuda) which is a US subsidiary of Nippon Shinyaku.

Nippon Shinyaku has been working actively towards their mission to develop agents for the treatment of intractable and rare c  
Their goal is to expedite the launch of products for patients with rare and unmet needs. With the Fast Track Designation, we r  
expedite development and promptly provide NS-065/NCNP-01 to DMD patients.

※1 NS-065/NCNP-01 is a morpholino antisense oligonucleotide, which is expected to improve muscle function for DMD patie  
amenable to exon 53 skipping. It was co-discovered by Nippon Shinyaku and National Center of Neurology and Psychiatry (N  
Kodaira City, Tokyo; President, Hidehiro Mizusawa, Director General, National Institute of Neuroscience, Shin'ichi Takeda) and  
drug candidate which is expected to be an important option for DMD.

※2 "SAKIGAKE designation (Japanese version of Breakthrough Therapy Designation) " is to promote R&D in Japan aiming a  
practical application for world's first, domestically-produced and innovative pharmaceutical products for serious and life-threat  
diseases. This system is aiming to shorten a reviewing time for approval, facilitating a prioritized consultation and review for r  
approval.

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